CIRM-Funded Study Helping Babies Battle a Deadly Immune Disorder Gets Boost from FDA

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Oakland, CA – A stem cell therapy that is helping save the lives of children born with a deadly immune disorder has been granted fast-track review status by the US Food and Drug Administration (FDA).

The California Institute for Regenerative Medicine (CIRM) has invested $12 million to test this therapy in a clinical trial at UC San Francisco.

The disorder is Artemis-SCID, a form of severe combined immunodeficiency disease. Children born with this condition have no functioning immune system so even a simple infection can prove life-threatening or fatal.

Currently, the only approved treatment for Artemis-SCID is a bone-marrow transplant, but many children are unable to find a healthy matched donor for that procedure. Even when they do find a donor they often need regular injections of immunoglobulin to boost their immune system.

In this clinical trial, UCSF Doctors Mort Cowan and Jennifer Puck are using the patient's own blood stem cells, taken from their bone marrow. In the lab, the cells are modified to correct the genetic mutation that causes Artemis-SCID and then re-infused back into the patients. The goal is that over the course of several months these cells will create a new blood supply, one that is free of Artemis-SCID, and that will in turn help repair the child's immune system.

So far the team has treated ten newly-diagnosed infants and three older children who failed transplants. Dr. Cowan says early data from the trial is encouraging. "With gene therapy, we are seeing these babies getting older. They have normal T-cell immunity and are getting immunized and vaccinated. You wouldn't know they had any sort of condition if you met them; it's very heartening."

Because of that encouraging data, the FDA is granting this approach Regenerative Medicine Advanced Therapy (RMAT) designation. RMAT is a fast-track designation that can help speed up the development, review and potential approval of treatments for serious or life-threatening diseases.

"This is great news for the team at UCSF and in particular for the children and families affected by Artemis-SCID," says Dr. Maria T. Millan, the President and CEO of CIRM. "The RMAT designation means that innovative forms of cell and gene therapies like this one may be able to accelerate their route to full approval by the FDA and be available to all the patients who need it."

Dr. Puck said the first child to receive the new stem cell treatment was born in a remote part of the Navajo nation, with no running water. "He was the first to get this space-age gene therapy, and he's now back on the reservation, four years old and running around in cowboy boots."

About CIRM

At CIRM, we never forget that we were created by the people of California to accelerate stem cell treatments to patients with unmet medical needs, and act with a sense of urgency to succeed in that mission.

To meet this challenge, our team of highly trained and experienced professionals actively partners with both academia and industry in a hands-on, entrepreneurial environment to fast track the development of today's most promising stem cell technologies.

With $5.5 billion in funding and more than 150 active stem cell programs in our portfolio, CIRM is the world's largest institution dedicated to helping people by bringing the future of cellular medicine closer to reality.

For more information go to www.cirm.ca.gov

About UCSF: The University of California, San Francisco (UCSF) is exclusively focused on the health sciences and is dedicated to promoting health worldwide through advanced biomedical research, graduate-level education in the life sciences and health professions, and excellence in patient care. UCSF Health, which serves as UCSF's primary academic medical center, includes top-ranked specialty hospitals and other clinical programs, and has affiliations throughout the Bay Area. Learn more at https://www.ucsf.edu, or see our Fact Sheet.

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